

Retinal progenitor cells for treatment of retinitis pigmentosa

Grant Award Details

Retinal progenitor cells for treatment of retinitis pigmentosa

Grant Type: Disease Team Therapy Development - Research

Grant Number: DR2A-05739

Project Objective: Retinitis pigmentosa (RP) is a severe form of rare blindness disease, yet represents an attainable near term target for stem cell therapy for a number of reasons. The therapeutic approach pursued by by this project is to save the light sensing cells of the eye (rod and cone photoreceptors) by transplanting a RPC type of stem cell known as a progenitor cell. These cells are capable of saving photoreceptors from degeneration following transplantation to the eye. These same cells are also highly efficient at producing new rod photoreceptors and this provides another more sustained pathway by which they preserve the crucial cone photoreceptors. In addition, there is evidence that the stem cells themselves might become functional photoreceptors and thereby stabilize the retina by directly replacing the dying cells in the patient's eye. Thus, transplanted stem cells could treat the targeted disease of RP in multiple ways simultaneously.

Investigator:

Name:	Henry Klassen
Institution:	University of California, Irvine
Type:	PI

Disease Focus: Vision Loss, Retinitis Pigmentosa

Human Stem Cell Use: Adult Stem Cell

Cell Line Generation: Embryonic Stem Cell

Award Value: \$17,144,825

Status: Active

Progress Reports

Reporting Period: Year 1

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Reporting Period:	Year 4
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Reporting Period:	Year 5/NCE
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Grant Application Details

Application Title: Retinal progenitor cells for treatment of retinitis pigmentosa

Public Abstract: The targeted disease is retinitis pigmentosa (RP), a severe form of blindness that often runs in families, but other times arises spontaneously from genetic errors. This disease is not overly common, yet represents an attainable near term target for stem cell therapy for a number of reasons: 1) RP destroys the light detecting cells of the retina in the back of the eye, yet generally leaves the rest of the visual system and body unharmed, so the clinical goal is circumscribed; 2) RP is prototypical of degenerations of the nervous system, so a cure for this less common disease would accelerate progress towards new therapies for a range of more familiar conditions; 3) scientific research has shown that degenerating rods and cones can be saved in animals by transplanting particular types of stem cells, thus the scientific feasibility of treating RP in this way has already been established in principle.

The therapeutic approach to be championed here is to save the light sensing cells of the eye (rod and cone photoreceptors) in people going blind using a type of stem cell obtained from the immature retina, but not from early embryos. These particular stem cells from the retina, known as progenitor cells, are capable of saving photoreceptors from degeneration following transplantation to the eye. These same cells are also highly efficient at producing new rod photoreceptors and this provides another more sustained pathway by which they preserve the crucial cone photoreceptors. In addition, there is evidence that the stem cells themselves might become functional photoreceptors and thereby stabilize the retina by directly replacing the dying cells in the patient's eye. Thus, transplanted stem cells could treat the targeted disease of RP in multiple ways simultaneously. Importantly, there are a host of reasons why clinical trials in the eye are easier and safer than most locations in the body. The eye is an important proving ground for stem cell-based therapies and provides a stepping stone to many otherwise incurable diseases of the brain and spinal cord.

As part of the current project, our cell type of interest will be grown under conditions ensuring pharmaceutical standards are met. The resulting cells will be tested in animals for safety and to make certain that they are therapeutically potent. An application will be made to the FDA seeking approval for the use of these cells in early clinical trials. Following approval, a small number of patients with severe RP will be injected with cells in their worse-seeing eye and followed clinically for a specified period of time to determine the safety and effectiveness of the treatment.

Statement of Benefit to California:

Benefits to the state of California and its citizens are both direct and indirect. The direct benefit is medical in that there is currently no cure or established treatment for the individuals and families that suffer from the dreadful hereditary blindness known as retinitis pigmentosa. In addition, there are many people in California and throughout the world that suffer from degenerative diseases of the retina, such as age-related macular degeneration (AMD), and the central nervous system that could benefit from further development and alternate applications of the type of stem cell therapy proposed in the current application. The rapid progress into the clinic that could be achieved via this proposal would help legitimize the use of stem cells for previously incurable diseases and should thereby accelerate the development of stem cell-based therapeutics for a wide range of other conditions. In so doing there would be an indirect benefit to California by making our state a focal point for stem cell breakthroughs. This would increase medical capabilities, strengthen the state's educational system, and energize local biotechnology companies with outside investment and a payoff in jobs and tax revenues. In the current economic situation, the citizens of the state are looking for a return on their investment in stem cell research. The leadership for the state is looking for a novel technology to reinvigorate the economy, provide desirable jobs, and reaffirm the flagship image of California. The project presented in this application has a real chance of provide that sort of spark.

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